

# (19) United States

# (12) Patent Application Publication (10) Pub. No.: US 2021/0332366 A1

Oct. 28, 2021 (43) Pub. Date:

#### (54) NOVEL SMALL ACTIVATING RNA

(71) Applicant: Ractigen Therapeutics, Nantong City

Inventors: Longcheng LI, Nantong City (CN); Moorim KANG, Nantong City (CN); Robert F. PLACE, Nantong City (CN); Jiancheng WU, Nantong City (CN)

(21) Appl. No.: 16/755,299

(22) PCT Filed: Apr. 10, 2019

(86) PCT No.: PCT/CN2019/082149

§ 371 (c)(1),

(2) Date: Apr. 10, 2020

#### (30)Foreign Application Priority Data

Apr. 10, 2018 (CN) ...... 201810317366.X

#### **Publication Classification**

(51) **Int. Cl.** C12N 15/113 (2006.01)A61P 35/00 (2006.01) (52) U.S. Cl.

CPC ...... C12N 15/1135 (2013.01); C12N 2320/34 (2013.01); C12N 2310/34 (2013.01); A61P 35/00 (2018.01)

#### (57)ABSTRACT

saRNAs are provided in the present invention. The saRNAs are composed of a first oligonucleotide strand containing 17 to 30 nucleotides and a second oligonucleotide strand containing 17 to 30 nucleotides. Sequences of at least 15 nucleotides in length are complementary in the two oligonucleotide strands, and the unpaired terminal nucleotides form overhangs. The first oligonucleotide strand or the second oligonucleotide strand has more than 75% homology or complementarity with any continuous fragment of 16 to 35 nucleotides in length in the promoter of the target gene. The second oligonucleotide strand has an overhang composed of 1 to 4 nucleotides at 3' end. saRNAs of the present invention can upregulate target gene expression more effectively while reducing off-target effects.

### Specification includes a Sequence Listing.

## p21 promoter sequence (1 kb)

-1000 goaggagga aaagtoctot ottocaacta tagtoattto tttgotgoat gatotgagtt -940 aggicaccag acticistga goccoagitt occcagoagi giatacgggo taigiggga -880 gtattoagga gasagacaac toastogtoa aatootooco ttootggooa acaaagotgo -920 tqcaaccaca qqqatttott ctqttcaqqt qaqtqtaqqq tqtaqqqaqa ttqqttcaat -760 gbocaattot totgtttooc toggagatoag gttgccettt titggtagte totccaatto -700 octoetteed ggaageatgt gacaateaan aactttgtat acttaagtte agtggacete -640 astituctus tetutgaset easugggant geaseatust totuguntus agetyotitu -580 ftggggfgta taggfgatos aggfgattat gggagaggfg acctagfgag ggataagfgg -520 gaatagaggt gatattgigg ggottitteig gaaattgoag agaggtgoat ogttittata -460 attratquat tittatqtat taatqtcatc cicciqatci titcaqciqc attqqqtaas -400 teettgeetg weagagtggg twageggtga gweagaaagg gggetwatte taacagtget -340 gtgtcctcct ggagagtgcc aactcattct coaagtaaaa aaagccagat ttgtggctca -280 etteqtqqqq aaatqtqtoo aqeqeaceaa eqeaqqqaaq qqaetqqqqq aqqaqaaq -220 typocetosty caybacycya gyttocyyya cpyyctyyco typtygaact byyddagypt -160 cagotgqoto gqcqotqqqu aqocaqqaqc otqqqoccoq qqqaqqqcqq toccqqqcqq -100 cycgologgic coagogogog tocogoctoc tiqaaqoggy cocqueogg gogqtiqtat -40 atcagggccg cgctgagotg cgccagctga ggtgtgagca gct